

Preclinical and clinical testing of a stem cell-based combination product for insulin-dependent diabetes

Grant Award Details

Preclinical and clinical testing of a stem cell-based combination product for insulin-dependent diabetes

Grant Type: Strategic Partnership I

Grant Number: SP1-06513

Project Objective: The primary objective of this award is to conduct a prospective, multi center, open-label, First in human Phase 1/2 study to evaluate the safety, tolerability and efficacy of the VC-01 Combination Product in adult subjects with Type 1 Diabetes Mellitus.

The VC-01 product is comprised of the PEC-01 pancreatic endoderm cells derived from hESC which are seeded onto the Encaptra drug delivery system, a cell-impermeable removable macro encapsulation device. The combination product VC-01-250 are implanted in the subcutaneous space of trial subjects for dose finding and the smaller VC-01-20 products are implanted in parallel in these subjects, to serve as sentinel devices, explanted at various time points and examined ex vivo.

Cohort 1 subjects (between 3-6 pts.) will be assessed for safety and tolerability and safety data collected from Cohort 1 will be evaluated by the DSMB to proceed with Cohort 2 (36 subjects). Efficacy endpoints, include stimulated C-peptide measurements following a MTT at 26 wks, changes in required insulin and glycemic control.

Investigator:

Name:	Howard Foyt
Institution:	ViaCyte, Inc.
Type:	PI

Disease Focus: Diabetes

Human Stem Cell Use: Embryonic Stem Cell

Award Value: \$9,475,070

Status: Active

Progress Reports

Reporting Period: Year 1

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Reporting Period:	Year 3
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Reporting Period:	Year 4
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Grant Application Details

Application Title:	Preclinical and clinical testing of a stem cell-based combination product for insulin-dependent diabetes
Public Abstract:	<p>Diabetes exacts a tremendous toll on patients, their families, and society. Autoimmune Type 1 diabetes, often called juvenile-onset diabetes, is caused by a person's own immune system mistakenly destroying their insulin-producing cells in the pancreas, known as beta cells. When those beta cells are lost, the ability to produce insulin in response to consumed carbohydrates is lost, and blood sugar can increase to toxic levels. Although not due to autoimmunity, Type 2 diabetics often lose their ability to produce insulin as well. While pharmaceutical insulin is commonly used to control both types of diabetes, it is difficult to self-administer optimally, does not sufficiently replace beta cells, and the adverse short- and long-term effects of diabetes and risks associated with insulin usage remain, including potentially fatal hypoglycemic episodes, nerve damage, blindness, kidney failure, foot ulcers / amputations, and heart disease.</p> <p>Ideally, one would like to replace lost beta cells, and attempts to do so have included the use of pancreas transplants, beta cell (islet) transplants, and transplants of animal cells. Unfortunately, those approaches are hindered by 1) a limited amount of donor tissue, and 2) issues regarding immunological incompatibility between donors and recipients. To solve the first problem, the group applying for this CIRM award has developed methods to make replacement beta cells from human embryonic stem cells (hESC), which can be reliably grown in large-scale batches. The hESC-derived beta cells have been shown to cure experimental diabetes in mice and rats. Regarding the issue of donor-recipient compatibility, the group has found that the cells can be administered under the skin in a simple device, essentially an envelope made of semi-permeable membrane, which is intended to protect the implanted cells from the patient's immune system. Upon implant, the cell-loaded device, which also keeps the implanted cells in place, acquires its own dedicated circulation. This blood supply provides oxygen and nutrients to the implanted cells, and also allows them to respond to blood sugar by releasing pancreatic hormones such as insulin into the circulation. Thus, the implanted cell-loaded device in essence represents a "replacement endocrine pancreas" with its own protection from autoimmunity. This product could return a patient's blood sugar regulation to normal and alleviate both the day-to-day and long-term issues of diabetes.</p> <p>The group has made tremendous progress in moving the product from concept through years of research and development. At this point an array of detailed work on the exact format to be tested in humans needs to be completed and submitted to the FDA on the way to clinical trials. The proposed award would provide critical funding, including potentially triggering matching funding from a large corporate partner, to advance the product through the first-in-human testing which will be very informative.</p>

**Statement of Benefit to
California:**

Diabetes mellitus currently afflicts approximately 350 million people worldwide, with projections of over 500 million by the year 2030 (sources: World Health Organization; International Diabetes Federation). In the year 2000 there were an estimated 2,089,657 cases of diabetes in California (diagnosed + undiagnosed; source: Diabetes Control Program, California Department of Health Services). Further, the disease disproportionately affects certain minority groups and the elderly. Despite the use of insulin and advances in its delivery, the human cost of diabetes is underscored by the financial costs to society: tens of billions of dollars each year in California alone. The primary cause of Type 1 diabetes, and contributing significantly to Type 2 diabetes as well, is the loss of insulin-producing pancreatic beta cells. The proposed Partnership will develop a beta cell replacement therapy for insulin-dependent diabetes. If successful, the therapy will go beyond insulin function, and will perform the full array of normal beta cell functions, including responding in a more physiological manner than manual or mechanized insulin administration. Because they will be more physiological, the replacement cells should also reduce the long-term effects of diabetes. Moreover, the cell therapy will alleviate patients of the constant monitoring of blood glucose and painful insulin injections. For these reasons, it is possible that the product could transform the diabetes treatment landscape dramatically and even replace pharmaceutical insulin in the market. This product will be available in California first, through clinical testing, and if approved by the FDA for commercial production, will eventually help hundreds of thousands of Californians with diabetes. The product will substantially increase quality of life for patients and their families while significantly reducing the health care burden in the state. The proposed Partnership will employ Californian doctors and scientists, and success will generate accolades and notoriety for the state. Lastly, once commercially marketed, the product will yield additional jobs in manufacturing, sales, and related industries, and generate revenue for California. Given the market need and the clear feasibility, the product could become the most significant stem cell-based medical treatment of the coming decade, and that will be a great achievement for California, its taxpayers, and CIRM.

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